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Reduced-Intensity Stem Cell Allografting for PNH Patients in the Eculizumab Era: The Mexican Experience

Alejandro Schcolnik-Cabrera [1], *Nancy Labastida-Mercado* [2,3], *Samantha L. Galindo-Becerra* [2,3], *David Gomez-Almaguer* [4], *Miguel A. Herrera-Rojas* [4], ***Guillermo J. Ruiz-Delgado*** [2,3,5], Guillermo J. Ruiz-Arguelles [5,6,7]. [1] *Universidad Nacional Autonoma de Mexico, Mexico City, Mexico;* [2] *Centro de Hematologia y Medicina Interna, Clinica Ruiz de Puebla, Puebla, Mexico;* [3] *Universidad Popular Autonoma del Estado de Puebla, Puebla, Mexico;* [4] *Hematology Service, University Hospital of Monterrey, Monterrey, Mexico;* [5] *Laboratorios Clinicos de Puebla, Clinica Ruiz, Puebla, Mexico;* [6] *Centro de Hematologia y Medicina Interna, Clinica Ruiz, Puebla, Mexico;* [7] *Universidad de las Americas Puebla, Puebla, Mexico*

Background: Paroxysmal nocturnal haemoglobinuria (PNH) presents as two major entities: the classical form, predominantly hemolytic, and a secondary type with marrow failure and resultant aplastic anaemia (AA-PNH). Currently, the treatment of choice of the hemolytic variant is eculizumab; however, the most frequent form of PNH in México is AA-PNH.

Patients and Methods: Six consecutive AA-PNH patients with HLA-identical siblings were allografted in two institutions in México, employing a reduced-intensity conditioning regimen for stem cell transplantation (RIST) conducted on an outpatient basis.

Results: Median age of the patients was 37 years (range 25–48). The patients were given a median of 5.4 $\times$ 10^6/kg allogeneic CD34(+) cells, using 1–3 apheresis procedures. Median time to achieve above 0.5 $\times$ 10^9/l granulocytes was 21 days, whereas median time to achieve above 20 $\times$ 10^9/l platelets was 17 days. Five patients are alive for 330–3150 days (median 1437) after the allograft. The 3150-day overall survival is 83.3%, whereas median survival has not been reached, being above 3150 days.

Conclusion: We have shown that hypoplastic PNH patients can be allografted safely using RIST and that the long-term results are adequate, the cost–benefit ratio of this treatment being reasonable. Additional studies are needed to confirm the usefulness of RIST in the treatment of AA-PNH.

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Ibrutinib Treatment of Relapsed CLL Following Allogeneic Transplantation: Sustained Disease Response and Promising Donor Immune Modulation

Christine E. Ryan [1], Aaron C. Logan [2], Andrew Rezvani [1], Manali Kamdar [1], Hideki Nakasone [1], Bita Sahaf [1], Joanne Otani [1], Katherine A. Kong [3], Mark Klinger [3], Malek Faham [3], Steven Coutre [4], David B. Miklos [1]. [1] *Division of Blood and Marrow Transplantation, Stanford University School of Medicine, Stanford, CA;* [2] *Helen Diller Family Comprehensive Cancer Center, University of California San Francisco, San Francisco, CA;* [3] *Sequenta, Inc., South San Francisco, CA;* [4] *Division of Hematology, Stanford University School of Medicine, Stanford, CA*

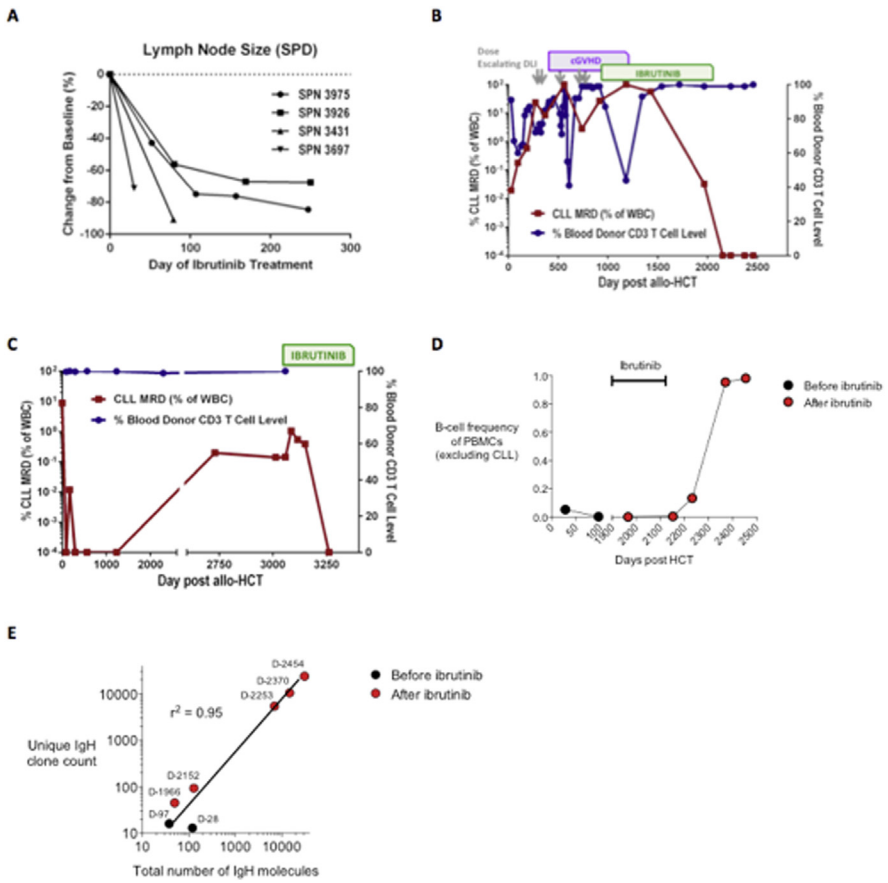

Figure 1. **(A)** Percent reduction in LN size, reported as sum of the product of LN diameters (SPD), for 4 patients following ibrutinib initiation. **(B)** CLL MRD (as percent of WBCs) and blood donor CD3 chimerism for SPN3975 and **(C)** SPN3431. **(D)** B cells (excluding the CLL clone) as percent of total PBMC for SPN3975. **(E)** Total IgH molecules and unique IgH clone counts for SPN3975 at time points (D=day) post allo-HCT.